

## WHAT IS CLAIMED IS:

1. A method of providing a therapeutic effect in a human patient, comprising:

administering to said patient CD34<sup>+</sup> cells obtained from cord blood, said CD34<sup>+</sup> cells having been genetically engineered to include at least one nucleic acid sequence encoding a therapeutic agent, thereby to provide said patient with said therapeutic agent by expression of said nucleic acid sequence in said patient.

2. The method of Claim 1 wherein said at least one nucleic acid sequence is contained in a viral vector.

3. The method of Claim 2 wherein said viral vector is a retroviral vector.

4. The method of Claim 1 wherein said therapeutic agent is adenosine deaminase.

5. The method of Claim 1 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5$ /kg to about  $10 \times 10^7$ /kg.

6. A method of treating a human patient suffering from severe combined immune deficiency resulting from adenosine deaminase deficiency, comprising:

administering to said patient CD34<sup>+</sup> cells obtained from cord blood, said CD34<sup>+</sup> cells having been genetically engineered to include a nucleic acid sequence encoding adenosine deaminase, said CD34<sup>+</sup> cells being administered to said patient in an amount effective to treat said adenosine deaminase deficiency in said patient by providing said patient with said adenosine deaminase by expression of said nucleic acid sequence encoding adenosine deaminase in said patient.

7. The method of Claim 6 wherein said nucleic acid sequence encoding adenosine deaminase is contained in a viral vector.

8. The method of Claim 7 wherein said viral vector is a retroviral vector.

9. The method of Claim 6 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5$ /kg to about  $10 \times 10^7$ /kg.

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Sub A2

10. The method of Claim 6 wherein said patient is a newborn infant and said CD34+ cells are obtained from the cord blood of said newborn infant.

*Sub a3*  
 11. A method of treating a human patient suffering from severe combined immune deficiency resulting from adenosine deaminase deficiency, comprising:

obtaining cord blood from an infant;  
 separating CD34+ cells from said cord blood;  
 cultivating CD34+ cells obtained from cord blood in the presence of (i) Interleukin-3; (ii) Interleukin-6; and (iii) a c-kit ligand;

transfecting said CD34+ cells with a nucleic acid sequence encoding adenosine deaminase; and

administering to said patient said transfected CD34+ cells, said CD34+ cells being administered to said patient in an amount effective to treat said adenosine deaminase deficiency in said patient by providing said patient with said adenosine deaminase by expression of said nucleic acid sequence encoding adenosine deaminase in said patient.

12. The method of Claim 11 wherein said nucleic acid sequence encoding adenosine deaminase is contained in a viral vector.

13. The method of Claim 12 wherein said viral vector is a retroviral vector.

14. The method of Claim 11 wherein said CD34+ cells are administered in an amount of from about  $5 \times 10^5$ /kg to about  $10 \times 10^7$ /kg.

15. The method of Claim 11 wherein said patient is a newborn infant and said CD34+ cells are obtained from the cord blood of said newborn infant.

*a* 16. A method of genetically engineering CD34+ cells obtained from cord blood with at least one nucleic acid sequence encoding a *therapeutic* therapeutic agent, comprising:

cultivating CD34+ cells obtained from cord blood in the presence of (i) Interleukin -3; (ii) Interleukin - 6; and (iii) a c-kit ligand; and

transfecting said CD34<sup>+</sup> cells with at least one nucleic acid sequence encoding a therapeutic agent.

17. The method of Claim 16 wherein said at least one nucleic acid sequence encodes adenosine deaminase.

18. The method of Claim 16 wherein said at least one nucleic acid sequence is contained in a viral vector.

19. The method of Claim 18 wherein said viral vector is a retroviral vector.

20. The method of Claim 18 wherein said CD34<sup>+</sup> cells are transduced at a multiplicity of infection of from 1 to 20 vectors per cell.

21. The method of Claim 5 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $1 \times 10^7/\text{kg}$ .

22. The method of Claim 21 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $5 \times 10^6/\text{kg}$ .

23. The method of Claim 9 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $1 \times 10^7/\text{kg}$ .

24. The method of Claim 23 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $5 \times 10^6/\text{kg}$ .

25. The method of Claim 14 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $5 \times 10^7/\text{kg}$ .

26. The method of Claim 25 wherein said CD34<sup>+</sup> cells are administered in an amount of from about  $5 \times 10^5/\text{kg}$  to about  $5 \times 10^6/\text{kg}$ .